CV4
A REVIEW OF PATIENT REGISTRIES IN HEART FAILURE ACROSS EUROPEAN UNION-5 COUNTRIES
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OBJECTIVES: Patient registries are a good source of evidence for health-care providers, payers and regulators requiring real-world data for evidence-based decision making. This review aimed to identify and assess key characteristics of the heart failure (HF) registries reporting patients' data from five European Union (EU5) countries. **METHODS:** HF registries were identified through a systematic search of Embase and PubMed databases. Inclusion criteria were data on HF patients from EU5 countries (France, Germany, Italy, Spain, and the UK) and patient counts ≥1000. Registry publications and websites were assessed for availability of information on patient characteristics, disease management, resource use, treatment outcomes and funding agency. **RESULTS:** Of 23 identified registries reporting data for HF patients, 43% were multinational while 57% were national-level registries. Twelve registries each collected data from Spain, Germany, and Italy and eight and six registries from France and the UK, respectively. Data was collected for patients with acute HF only (31%), chronic HF only (43%), and both acute and chronic HF (26%) in the identified registries. Number of included patients varied from 1037 to 25,000 with the average follow-up ranging from 3-53 months. Data was available for patient demographics (in 100% registries), comorbidities (87%), diagnosis and disease classification (78%), mortality (78%), hospitalisation (96%), prescribed drugs (100%), and patient-reported outcomes (17%). Registries also collected data on specific diagnostic tools (blood pressure, ECG, blood tests, biomarkers, imaging, and renal function tests), treatment procedures (revascularisation, catheterization, and transplant), resource use, adverse events, and cost. Of these registries, 65% initiated during last 10 years and 61% were industry-funded. **CONCLUSIONS:** Registries are a rich source of real-world information on HF patients which can be turned into actionable insights for important health-care decisions. Rise in the number of HF registries in recent years and industry funding indicates increased interest of stakeholders in these registries' data.

EQUITY & ACCESS STUDIES

EA1
HOW READY ARE EUROPEAN PAYERS FOR EMA ADAPTIVE PATHWAYS?
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OBJECTIVES: In April 2014, the European Medicines Agency (EMA) announced an adaptive pathways pilot enabling initial approval of a medicine for a restricted patient population with very high unmet need based on early clinical studies. Progressive adaptations of the marketing authorisation would expand access to broader patient populations based on subsequent data. However, such an adaptive license may pose a problem for payer bodies, many of which currently require a long time period to review medicines under strict clinical and economic criteria. This research aims to evaluate how ready major European current payer systems would be to encompass EMA adaptive pathways. **METHODS:** Key European payer bodies from the EU5 (NICE, SMC, HAS, IQWiG/G-BA, AIFA and AEMPS were scored by PAREXEL's Commercialization panel of pricing and reimbursement experts on 4 key criteria: (1) speed of appraisal, (2) flexibility to clinical data appraisal approach, (3) flexibility to economic data appraisal approach, and (4) no additional local/regional access hurdles as either high (2 points), medium (1 point) or low (0 points). **RESULTS:** Out of a possible 8 points, HAS scored the highest (7) followed by SMC (6), AIFA (5), AEMPS (5), IQWiG (4) with NICE the lowest (2). Low scores for NICE were driven by its long duration for decision-making alongside rigid adherence to ICER thresholds. Higher scores for SMC were based on their much faster appraisals and flexibility through the new PACE process. AIFA and AEMPS both scored moderately with positive flexibility in their approaches tempered by additional local/regional access hurdles. In Germany, rigid adherence to direct comparative trial data versus the relevant comparator limited positive scoring. **CONCLUSIONS:** some payers (most notably NICE) will need to make significant changes to their appraisal processes In order for early market approval under adaptive pathways to translate into early reimbursement and payer access

EA2
ACCESS TO INNOVATIVE DRUGS IN PATIENTS WITH METASTATIC LUNG CANCER IN FRENCH PUBLIC HOSPITALS (THE TERRITOIRE STUDY)
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OBJECTIVES: Lung cancer survival is socioeconomically patterned, and socioeconomic inequalities in receipt of treatment have been demonstrated in several countries. In the hospitals, many innovative anticancer drugs are too expensive to be funded through a Diagnosis-Related Group (DRG) of chemotherapy administration. In France, such drugs are fully reimbursed up to national reimbursement tariffs (extra-DRG funding) to ensure equity of access. Our aim was to analyze the access of patients to innovative drugs according to social deprivation index. **METHODS:** A retrospective cohort study was constituted with all patients having a diagnosis of metastatic lung cancer in the French National hospitals databases (PMSI) during year 2011. Patients' data were linked to allow a two-year follow-up period. Because extra-DRG data were not available for private hospitals, our analysis was restricted to patients benefiting from chemotherapy in public hospitals only. In addition of demographic characteristics, comorbidities, and treatment, we assigned each patient to social deprivation index based on their postcode of residence. **RESULTS:** We identified 11,602 patients receiving chemotherapy in public hospitals. During follow-up, 7,417 patients (63.9%) received expensive drugs at least once, including mostly pemetrexed (57.5%), bevacizumab (16.9%), or topotecan (7.2%); these patients were significantly more likely women and younger than the rest of the cohort (p<0.0001). Conversely, all selected comorbidities were associated with lower rates of administration i.e. chronic renal failure, diabetes, hypertension, COPD and other respiratory diseases (p<0.0001). Taking as reference patients from affluent areas, we observed lower rates of access in intermediary affluent, intermediary deprived and deprived areas. After multivariate adjustment, odd ratios were 0.85 [95%:0.75–0.95], 0.82 [95%:0.74–0.92] and 0.80 [95%:0.71–0.89], respectively. **CONCLUSIONS:** Even in a health care system organized to ensure a high degree of equity in medical care, we found indications of a socioeconomic gradient in innovative anticancer drugs access in lung cancer.

EA3
THE ECONOMIC IMPACT OF AN HYPOTHETICAL RX-TO-OTC SWITCH IN SPAIN
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OBJECTIVES: Tight public budget constraints have accelerated the debate on the extent of prescribed medications that are publicly covered in Spain, a country with one of the lowest percentage of OTC sales over total drugs sales. The objective of this study is to examine the economic impact of payer policies consisting on a hypothetical Rx-to-OTC switch of 5% of currently publicly covered prescription drugs for mildest conditions. **METHODS:** A decision model is used to estimate the economic impact of the switch. Both third-party-payer and societal perspectives are adopted, using a five-year timeframe, both at regional (17 regions) and national levels. The model is inspired by a previous ones in European countries, but the timeframe and the effects measured have been further developed for the sake of robustness and better reflection of the current Spanish reality. The effects, measured in monetary units, include those regarding to quality of primary care, labor productivity, the opportunity cost for non-working populations, and financial burden of pharmaceutical expenditure. Resources allocated to improving responsible self medication are also accounted for. The study analyses the effects on different stakeholders (patients, NHS, and society). Finally, it includes both a standard and a probabilistic sensitivity analysis. **RESULTS:** The switch results in a 3.125€ millon societal effect, explained by improving quality of primary health care (2.258M€), improving labor productivity (507), optimization of non working patients'time (579) and a reduction of public expenditure on publicly covered medication (979). On the other hand, there are negative effects from increasing privately funded drugs (1.165) and the cost of improving self-care (35). Sensitivity analyses confirm overall results (p<0,01). **CONCLUSIONS:** Under increasing budgets constraints, switching drugs for mild conditions may be a solution with positive societal net effects, although the distribution of the impacts among stakeholders might make the initiative unpopular.

EA4
ORPHAN DESIGNATIONS AND APPROVALS IN THE EU, UNITED STATES AND JAPAN
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OBJECTIVES: Since the introduction of orphan legislation the number of orphan drugs has dramatically increased. The objective of the study is to compare orphan designations and approvals in the EU, USA and Japan. **METHODS:** The list of molecules with orphan status, as well as the list of approved orphan drugs (ODs) in the USA, EU and Japan were identified from the websites of Food and Drug Administration (FDA) of the USA, the European Medicines Agency (EMA), and National Institute of Biomedical Innovation, Health and Nutrition. All available details on orphan designations and approvals were extracted. **RESULTS:** By June 2015, 3465 orphan designations were granted in the USA, 1503 in the EU and 399 in Japan. More than 60% of orphan designations led to an approval in Japan, about 14% in the USA, and only 7% in the EU. The median delay between granting an orphan designation and approval was more than three years in the USA and EU, and about two years in Japan. EMA publishes the most detailed information on orphan designations and OD approvals. Unlike the USA and Japan, the details on refusals in orphan status or approvals together with decision rationale are available in the EU. **CONCLUSIONS:** A higher number of ODs has been approved in the USA. However, many of them have been discontinued, particularly among ODs approved before 2000. The relatively small number of ODs approved in the EU might be related to the lower financial support of ODs provided in the EU compared to the USA and Japan. A noticeably high commercialisation rate is observed in Japan, which can be associated with the feasibility criteria applied to the drugs submitting for orphan designation on the one hand, and with the arrival on Japan market of molecules already commercialized in other countries on the other.

HEALTH TECHNOLOGY ASSESSMENT STUDIES

HT1
ACCESS TO NEW THERAPIES IN ROMANIA THROUGH THE SCORECARD HTA SYSTEM
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OBJECTIVES: The objectives are to present the scorecard HTA system used in Romania from July 2014 and the results in terms of access of new therapies at reimbursement. **METHODS:** The authors studied the scorecard HTA legislation and the relationship with the health-care environment from the last 2 years considering: the evolution of the Romanian HTA methodologies, the HTA process and the implications in other